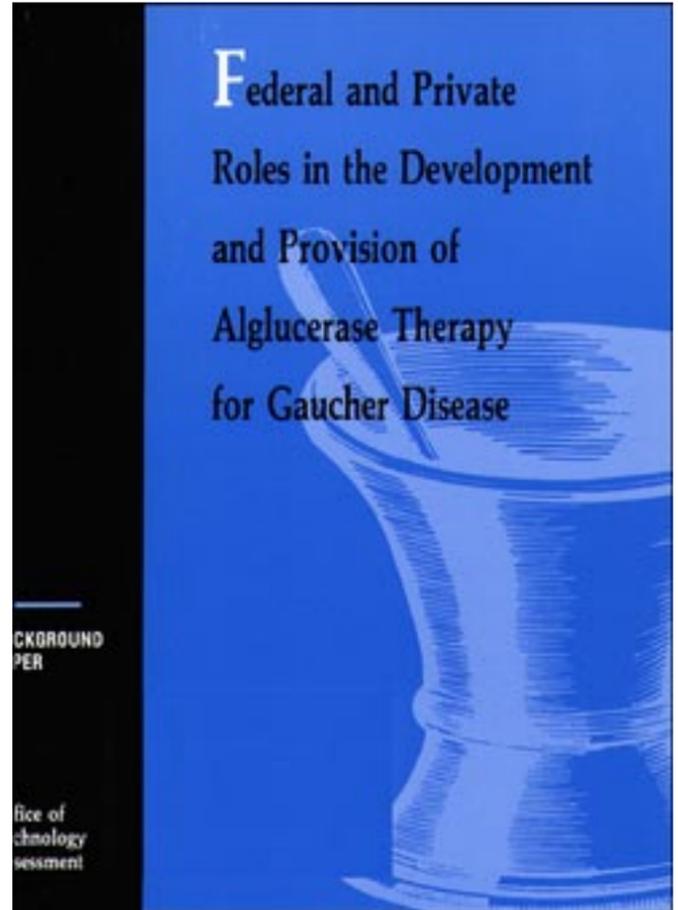


*Federal and Private Roles in the  
Development and Provision of Alglucerase  
Therapy for Gaucher Disease*

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# Foreword

The effort to discover and develop new pharmaceuticals is a risky and costly enterprise. For diseases that affect few patients, the barriers to development maybe especially great, since the drugs' small markets may make it difficult for firms to recoup their initial research and development investments. The Federal Government has sought to reduce these barriers through incentives first adopted in the Orphan Drug Act of 1983 (Public Law 97-414). The transfer of technology from Federal laboratories such as the National Institutes of Health to the pharmaceutical industry can also reduce the cost and risk of drug development for firms. Although such incentives may result in important new therapies, their price to patients and insurers may still be high.

As part of our assessment, *Government Policies and Pharmaceutical Research and Development*, requested by the House Committee on Energy and Commerce and its Subcommittee on Health and the Environment and the Subcommittee on Antitrust, Monopolies, and Business Rights of the Senate Committee on the Judiciary, OTA commissioned researchers at Stanford University to examine the development and provision of alglucerase, an important new treatment for Gaucher disease. Gaucher disease is a rare inherited disorder in which the body lacks an enzyme necessary to break down fats. This background paper describes the development of alglucerase, illustrates the role that both the Federal Government and private sector can have in making new therapies available for orphan diseases, and lays out some of the tradeoffs that can exist between developing new medical technologies and controlling health care costs.

**X<sup>A</sup>2&?** -  
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<sup>1</sup> From July 1991 to February 1992.

# **Federal and Private Roles in the Development and Provision of Alglucerase Therapy for Gaucher Disease**

## **Background Paper**

This background paper was prepared as part of OTA's assessment of Government Policies and Pharmaceutical Research and Development.

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## Glossary of Abbreviations

BCBS	—Blue Cross and Blue Shield	HMO	—health maintenance organization
CHAMPUS	--Civilian Health and Medical Program of the Uniformed Services	IND	—Investigational New Drug
DNA	-deoxyribonucleic acid	NDA	—New Drug Application
FDA	—Food and Drug Administration	NIH	—National Institutes of Health
HCFA	—Health Care Financing Administration	ODA	-Orphan Drug Act
HCPCS	—HCFA's Common Procedure Coding System	R&D	—research and development
		SSA	-Social Security Administration